2022 COVERAGE FOR SCD SUMMIT

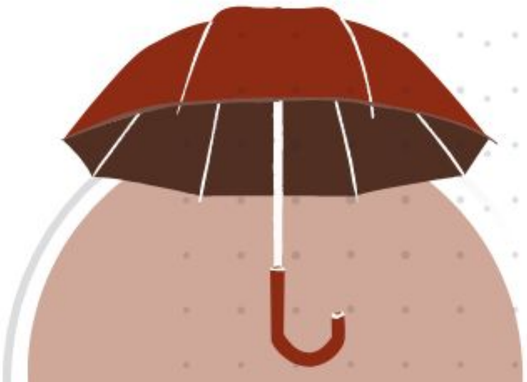
SICK CELLS

2022
COVERAGE FOR SCD SUMMIT

Best Practices for Payer Management of Sickle Cell Disease (SCD)

August 31, 2022
12 – 4 PM

*All Time are reflected in EDT



Welcome

Ashley Valentine, MRes
Co-Founder and President of Sick Cells

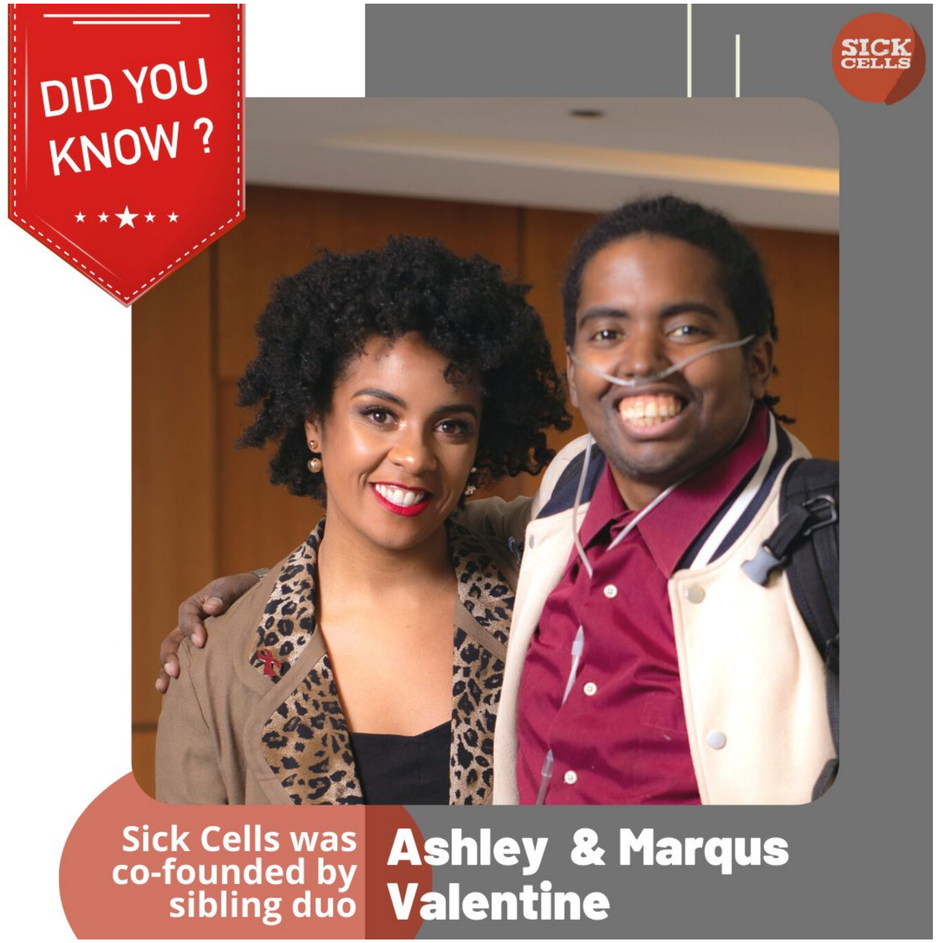
Meet the Valentines

Marqus and Ashley Valentine
Co-Founder of Sick Cells

Who We Are

Sick Cells is a sickle cell disease (SCD) advocacy nonprofit founded February 28, 2017.

Sick Cells' mission is to elevate the voices of the SCD community and their stories of resilience. In highlighting the grave disparities this community faces, we hope to influence decision-makers and propel change.



Who We Are

Sick Cells seeks to **elevate the voices** of the sickle cell disease (SCD) community and their stories of resilience.

Ignite

Humanize

Inspire

Influence

Drive

Empower

AGENDA

*All times listed in EDT

12:00 - 12:30 PM

OPENING & KEYNOTE

- Opening by Ashley Valentine
- Keynote presentation by Dr. Ahmar Zaidi

12:30 - 1:00 PM

THE STATE OF ACCESS IN MEDICAID FOR PRESCRIPTION DRUGS TREATING SICKLE CELL DISEASE

- Presentation by Maggie Jalowsky and Emma Andelson from Sick Cells

1:00 - 2:00 PM

IMPROVING EQUITY AND AFFORDABILITY OF SCD THERAPIES: BEST PRACTICES IN BENEFIT DESIGN & PAYER MANAGEMENT

- Faculty includes Francesca Valentine, Dr. Shivi Jain, Dr. Emily Tsiao, Dr. Terry Cothran, and Dr. Terry Richardson
- **CE/CME Accredited Session**

AGENDA
(CONTINUED)

*All times listed in EDT

2:00 - 3:00 PM

PATIENT JOURNEY SPOTLIGHT: WHAT DOES ACCESS AND COVERAGE LOOK LIKE FOR PATIENTS?

- Discussion by Khristina Reid, Rae Blaylark, Blaze Eppinger, and Chifuan Powell
- Followed by a 10-minute stretch break

3:00 - 4:00 PM

A MANAGED CARE APPROACH FOR SCD & CLOSING

- Presentations by John Stancil, Dr. John Watkins, and Chanell Grismore
- Moderation by Adrienne Shapiro
- Closing reflections by Ashley Valentine

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Bio Biotechnology Innovation Organization

2022 COVERAGE FOR SCD SUMMIT

SICK CELLS

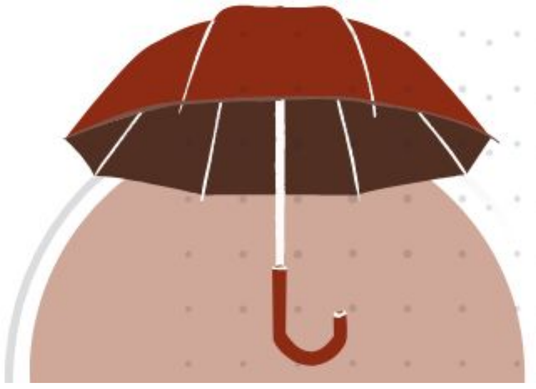
KEYNOTE PRESENTATION:

THE SICKLE CASTE: RE-HUMANIZING SICKLE CELL CARE TOGETHER

Dr. Ahmar U. Zaidi

Speaker: Ahmar U Zaidi, MD

Time: 12 - 12:25 PM EDT



the sickle caste:

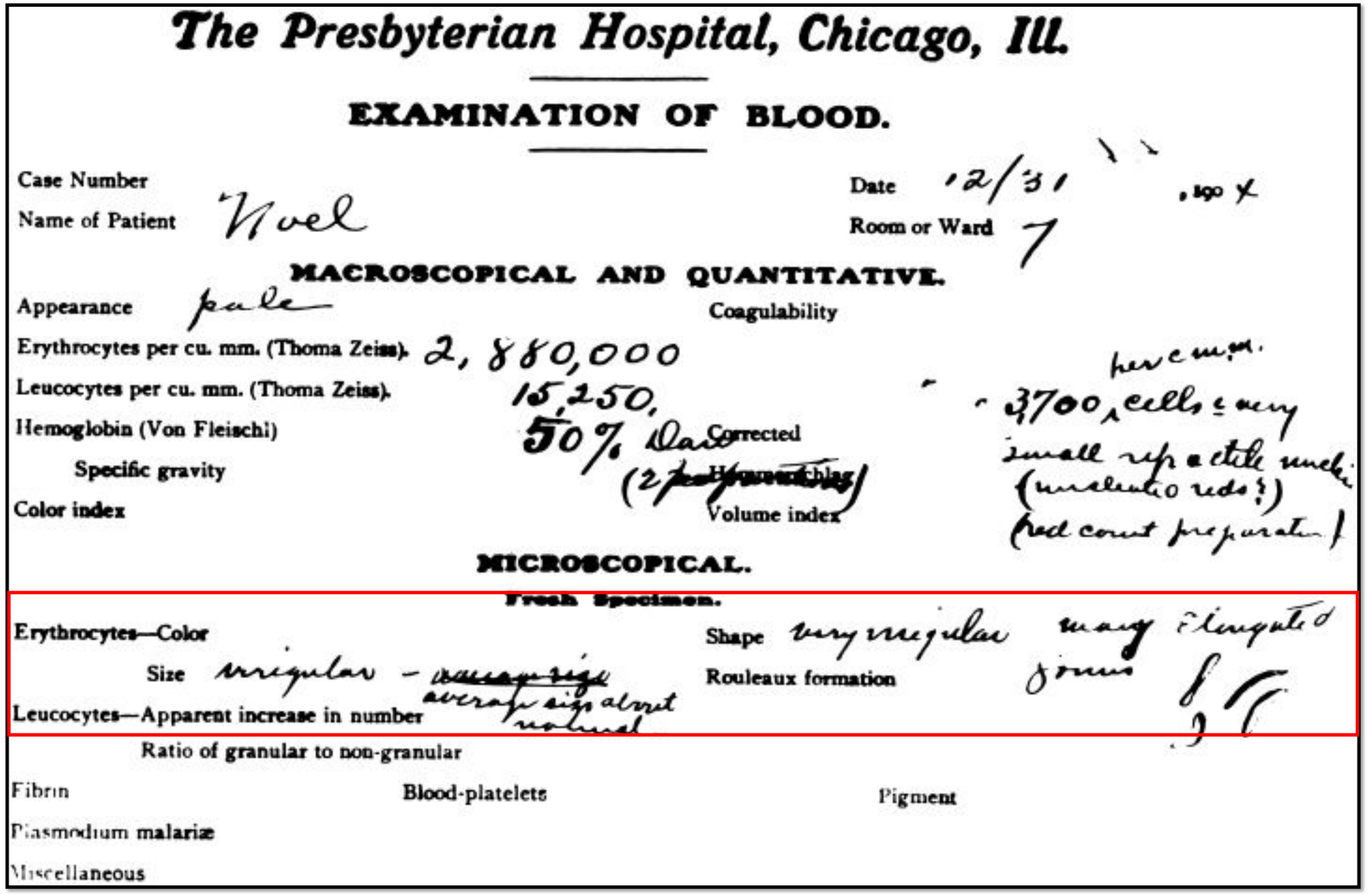
re-humanizing sickle cell care together

Ahmar U. Zaidi, MD
@drzsicklecell

Disclosures

- **Employment and Equity:**
  - **AUZ is an employee of Agios Pharmaceuticals, Inc.**
  - **The opinions and positions expressed in this presentation reflect AUZ's personal views and do not necessarily reflect the opinions or positions of Agios.**
- Advisory Boards/Honoraria:
  - Commercial: Global Blood Therapeutics, Novartis
  - Medical: Global Blood Therapeutics, Emmaus Life Sciences, Cyclerion, bluebird bio, Chiesi, NovoNordisk, GraphiteBio
- Research/Clinical Trial:
  - Imara, Forma Therapeutics, Global Blood Therapeutics
- Speaker Bureau:
  - Global Blood Therapeutics
- Funding:
  - Emmaus Life Sciences

1904: The SS Cearense



The Presbyterian Hospital, Chicago, Ill.

EXAMINATION OF BLOOD.

Case Number

Name of Patient Noel

Date 12/31 .190 X

Room or Ward 7

MACROSCOPICAL AND QUANTITATIVE.

Appearance pale

Coagulability

Erythrocytes per cu. mm. (Thoma Zeiss). 2,880,000

Leucocytes per cu. mm. (Thoma Zeiss). 15,250.

Hemoglobin (Von Fleischl) 50% Dale Corrected

Specific gravity

Color index

Volume index

per cu.mm. 3700, cells & very small reproductive nucleated reds?) (red count preparation)

MICROSCOPICAL.

Fresh Specimen.

Erythrocytes—Color

Size irregular – ~~macrocytic~~ average size about normal

Shape very irregular many elongated forms

Rouleaux formation

Leucocytes—Apparent increase in number

Ratio of granular to non-granular

Fibrin

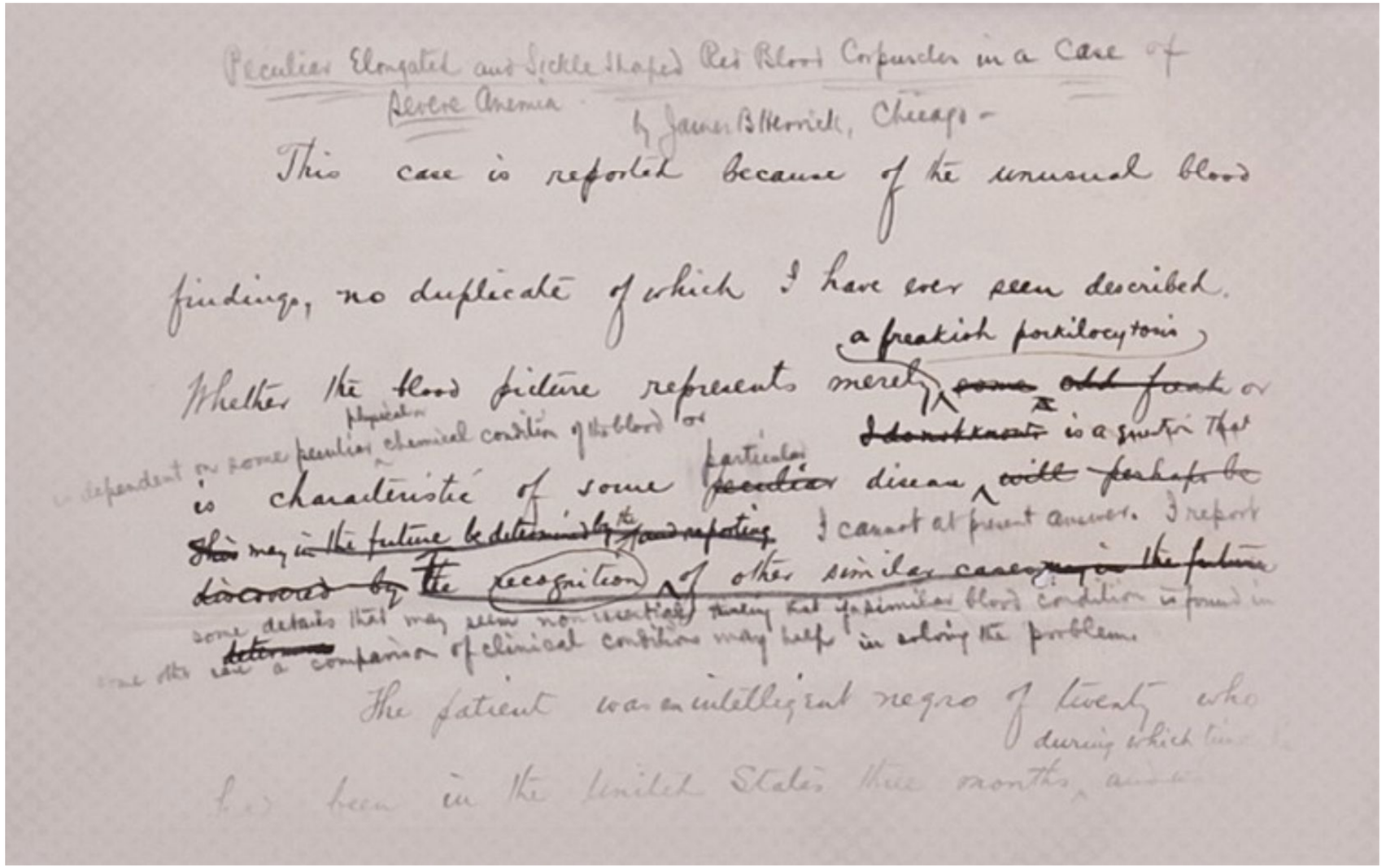
Blood-platelets

Pigment

Plasmodium malariæ

Miscellaneous

Walter Clement Noel



Peculiar Elongated and Sickle Shaped Red Blood Corpuscles in a Case of Severe Anemia.

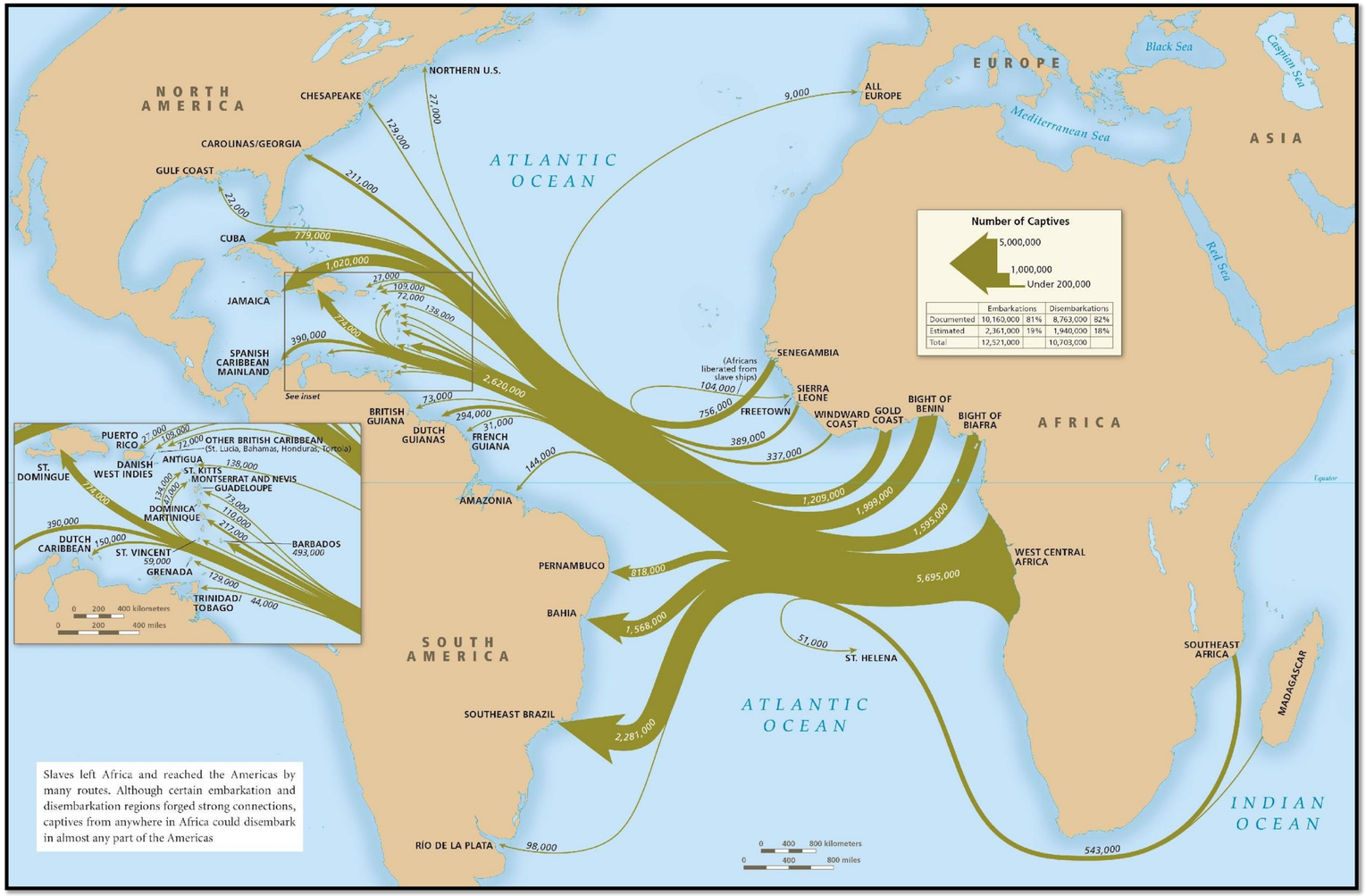
by James B. Herrick, Chicago.

This case is reported because of the unusual blood findings, no duplicate of which I have ever seen described.

Whether the blood picture represents merely a freakish poikilocytosis ~~some odd fact~~ or ~~I don't know~~ is a sign that is dependent on some peculiar physical chemical condition of the blood or is characteristic of some particular disease ~~will perhaps be~~ ~~this may in the future be determined by the recognition~~ I cannot at present answer. I report ~~discovered by the recognition~~ of other similar cases ~~in the future~~ some details that may seem non essential thinking that a similar blood condition is found in ~~determine~~ one the case a comparison of clinical conditions may help in solving the problem.

The patient was an intelligent negro of Texas who during which time he has been in the United States three months, and

1619: The Trans-Atlantic Slave Trade



“The Patients”

“All the good qualities of this tea,… praised as they are, **cannot however prevent the sickliness of the inhabitants, especially prevalent in the low, overflowed, and swampy parts of this country, and giving the people a pale, yellow, and prematurely old look…and visited with numerous fevers**…”

- Dr. Johann Schoepf in 1783 as he traveled through the Carolinas

“the poor, unfortunate man had leg ulcers and…an absent spleen.”

- Lebby, R. Case of Absence of the Spleen.

Southern Journal of Medical Pharmacology (1846)

There has been a systematic dehumanization of patients with sickle cell disease, within the confines of structural racism that they face.

We are all responsible for its persistence.

We will be responsible for its dismantling.

Disparity in every realm of health

CARDIOVASCULAR DISEASE

- 3x more likely to develop
- 2x more likely to die from
- less likely to receive treatment of choice
- less likely to get preventative measures
- more likely for early discharge

KIDNEY DISEASE

- black patients get more dialysis and less organ transplant

CANCER

- NCI: "when black people get equal care to white people, survival rates are equal"
- later diagnosis
- less treatment
- less intervention

Obesity and childhood obesity

- 48 percent of adults are **obese**.[3]
- High rates of severe **childhood obesity**.[4]

Diabetes

- 80 percent more likely to be **diagnosed** with diabetes.
- 2.4 times more likely to begin treatment for **end-stage renal disease.**[5]
- 1.7 times more likely to be **hospitalized**.[5]
- 20 percent more likely to have **visual impairments**.[5]

Heart disease

- Men are 30 percent and women 60 percent more likely to **have high blood pressure**.[6]
- Less likely to keep their **blood pressure under control**.[7]
- Men have twice the risk of first time **stroke**.[8]

Cancer

- Women are 40 percent more likely to die of **breast cancer**.[9]
- Men are 1.3 times more likely to have new cases of **colorectal cancer**.[9]

Maternal and child health

- Children are 1.8 times more likely to have ever been told they have **asthma**.[10]
- 3.5 times as likely to die as infants due to complications related to **low birthweight**.[11]
- 2.2 times higher **infant mortality** rate.[11]

Mental health

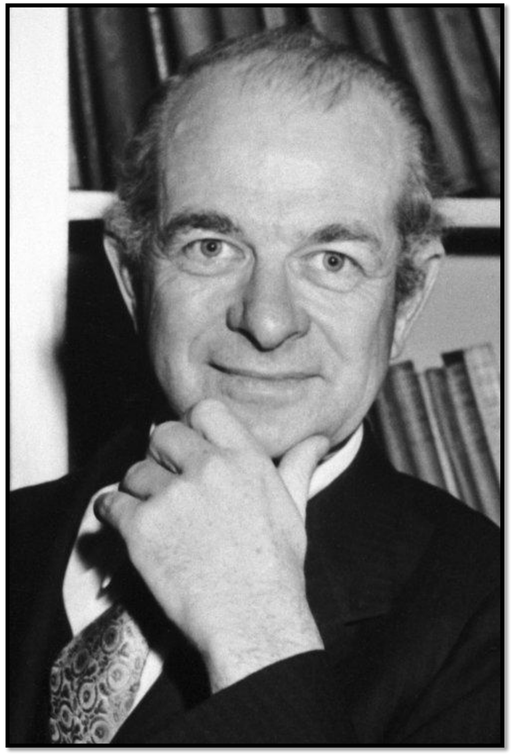
- 20 percent more likely to report **psychological distress**.[12]
- 50 percent less likely to receive counseling or **mental health treatment**.[12]

Vulnerable populations disproportionately affected by COVID-19

Risk for COVID-19 Infection, Hospitalization, and Death By Race/Ethnicity

Updated Mar. 25, 2022 Print

Rate ratios compared to White, Non-Hispanic persons	American Indian or Alaska Native, Non-Hispanic persons	Asian, Non-Hispanic persons	Black or African American, Non-Hispanic persons	Hispanic or Latino persons
Cases[1]	1.6x	0.7x	1.1x	1.5x
Hospitalization[2]	3.1x	0.8x	2.4x	2.3x
Death[3]	2.1x	0.8x	1.7x	1.8x



Winner of

Nobel Prize

in Chemistry

LINUS PAULING

of the California Institute of Technology

Lecturing on

"Abnormal Human Hemoglobin Molecules in Relation to Disease"

TUESDAY, NOVEMBER 6

8:15 P.M.

TODD AUDITORIUM - - - PULLMAN

Sponsored by: Washington-Idaho Border Section American Chemical Society

About 600 auditors

Sickle Cell Anemia, a Molecular Disease[1]

Linus Pauling, Harvey A. Itano,[2] S. J. Singer,[2] and Ibert C. Wells[3]

Gates and Crellin Laboratories of Chemistry,
California Institute of Technology, Pasadena, California[4]

THE ERYTHROCYTES of certain individuals possess the capacity to undergo reversible changes in shape in response to changes in the partial pressure of oxygen. When the oxygen pressure is lowered, these cells change their forms from the normal biconcave disk to crescent, holly wreath, and other forms. This process is known as sickling. About 8 percent of American Negroes possess this characteristic; usually they exhibit no pathological consequences ascribable to it. These people are said to have sicklemia, or sickle cell trait. However, about 1 in 40 (*4*) of these individuals whose cells are capable of sickling suffer from a severe chronic anemia resulting from excessive destruction of their erythrocytes; the term sickle cell anemia is applied to their condition.

that form from normal erythrocytes. In this condition they are termed promeniscocytes. The hemoglobin appears to be uniformly distributed and randomly oriented within normal cells and promeniscocytes, and no birefringence is observed. Both types of cells are very flexible. If the oxygen or carbon monoxide is removed, however, transforming the hemoglobin to the uncombined state, the promeniscocytes undergo sickling. The hemoglobin within the sickled cells appears to aggregate into one or more foci, and the cell membranes collapse. The cells become birefringent (*11*) and quite rigid. The addition of oxygen or carbon monoxide to these cells reverses these phenomena. Thus the physical effects just described depend on the state of combination of the hemoglobin, and only secondarily, if at all, on the cell membrane.

Bad Genes and Marriage

By BARBARA YUNCKER

Persons who carry the recessive genes of severe inheritable disease should probably not have children, two famed scientists advised today in order not to increase the bad-gene load of the race.

The two are Nobel laureates Sir Peter Medawar of London and Dr. Linus Pauling, who holds the prize both for chemistry and for peace efforts. They were in town to participate in the dedication of the new Mount Sinai School of Medicine and inauguration of its president and dean, Dr. George James.

Sir Peter, whose prize honored pioneer work in the understanding of immunology and tissue rejection, said two carriers of the same defective gene—for Cooley's anemia or the retardation deficiency PKU, for example—"should be discouraged from marrying each other" because half their children will be carriers and a quarter of their children will be victims.

"It is humbug to say that such a policy violates an elementary right of human beings," Medawar said. "No one has conferred upon human beings the right knowingly to bring maimed or biochemically crippled children into the world."

Genetic Inflation

Even with such limitations, he said, "The frequency of the malignant gene will steadily rise . . . We are dealing here with a genetic equivalent of inflationary economics; we seem to be getting on all right, but the currency is deteriorating."

Countries to this he said, could come through medical advances such as direct genetic repair which he called "not likely, but not inconceivable . . . or, more likely, very early embryonic diagnosis of gross derangements of the chromosomal apparatus"—presumably with therapeutic abortion.

Pauling, speaking later at the "Future of Medicine" session at City College, said: "I agree we should keep these carriers from marrying one another. I have advised, not entirely joking, that individuals should have tattooed on their foreheads swmbols for the defective genes they carry . . .

"Because of certain objections which might be raised, a ribald friend suggests it would be better to tattoo the symbols n Braille on their abdomens.

Carriers who marry normals, he said, can produce carriers, so they "have an obligation to produce a decreased number of children, at least."

Dr. Francis H. C. Crick of Cambridge, who won a Nobel for his share in deciphering the structure of DNA, the basic genetic material, predicted the new area of ferment in medicine would be in study of the nervous system.

"f all the things we know about man, compared to what we would like to know, the nervous system is the one we know least . . . It is complex and we are complex for that reason."

The fourth Nobelist on the panel, Dr. George W. Beadle, now president of the University of Chicago, who elucidated how genes direct production of enzymes, stressed social responsibility of medical schools, particularly in urban centers, as his own and Sinai are. He warned that "black separatism may counteract and even reverse the trend . . . to [help] disperse, dilute and cure the concentrated social and medical ills" of urban slum areas in cooperative programs with their residents.

James in his inaugural address stressed the need to "redefine the very concept of medical care. The tenfold higher tuberculosis rates in certain areas of our city do not mean that the tuberculosis organism is more virulent there or that our drugs are any less effective in such areas. It must be the poverty and all that accompanies this which is responsible."

Govc. Rockefeller, in closing the day-long celebration of the new school affiliated with the City University of New York, praised it as proof hat "private citizens and private instituion citizens and private initiative and private enterprise can do a large job on a large scale."

with therapeutic abortion.

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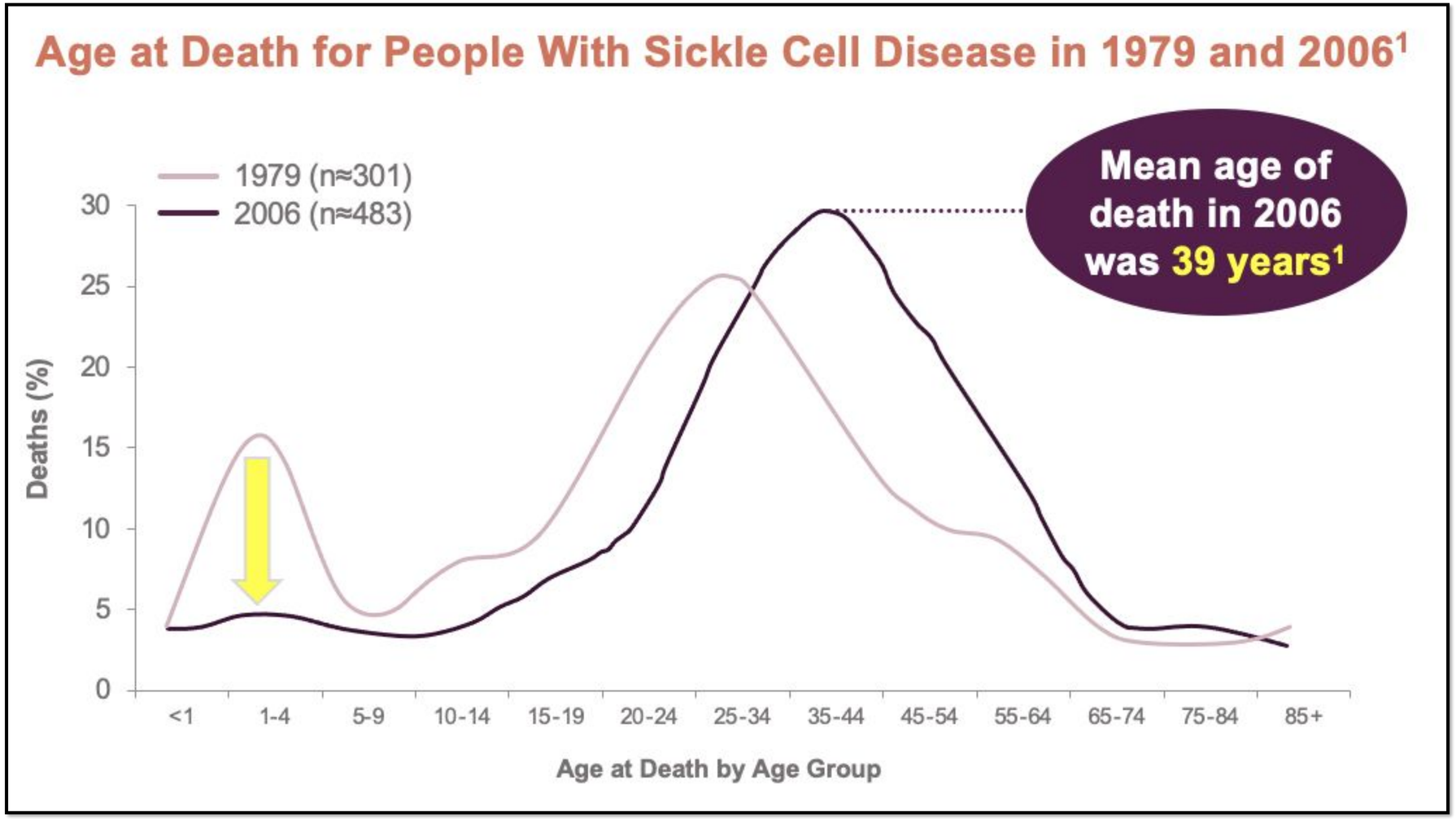
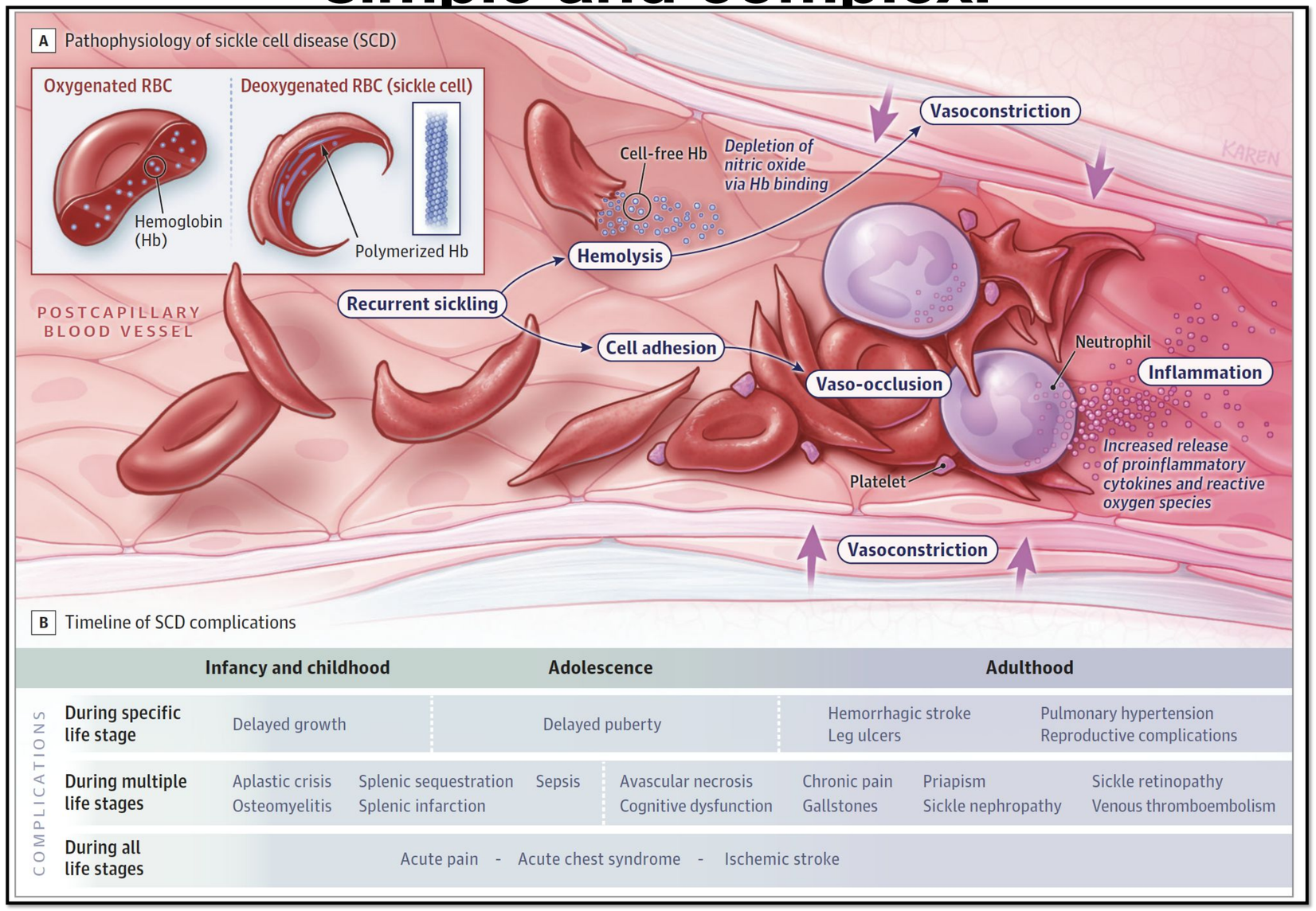
Predictable and heartless outcomes.

- Patients with sickle cell disease:
  - wait longer in emergency rooms
  - wait longer for their first medication
  - aren’t believed when reporting pain scores
  - are stigmatized and shunned
  - have disparate research funding when compared to other rare diseases
  - don’t have appropriate management of pain/disease

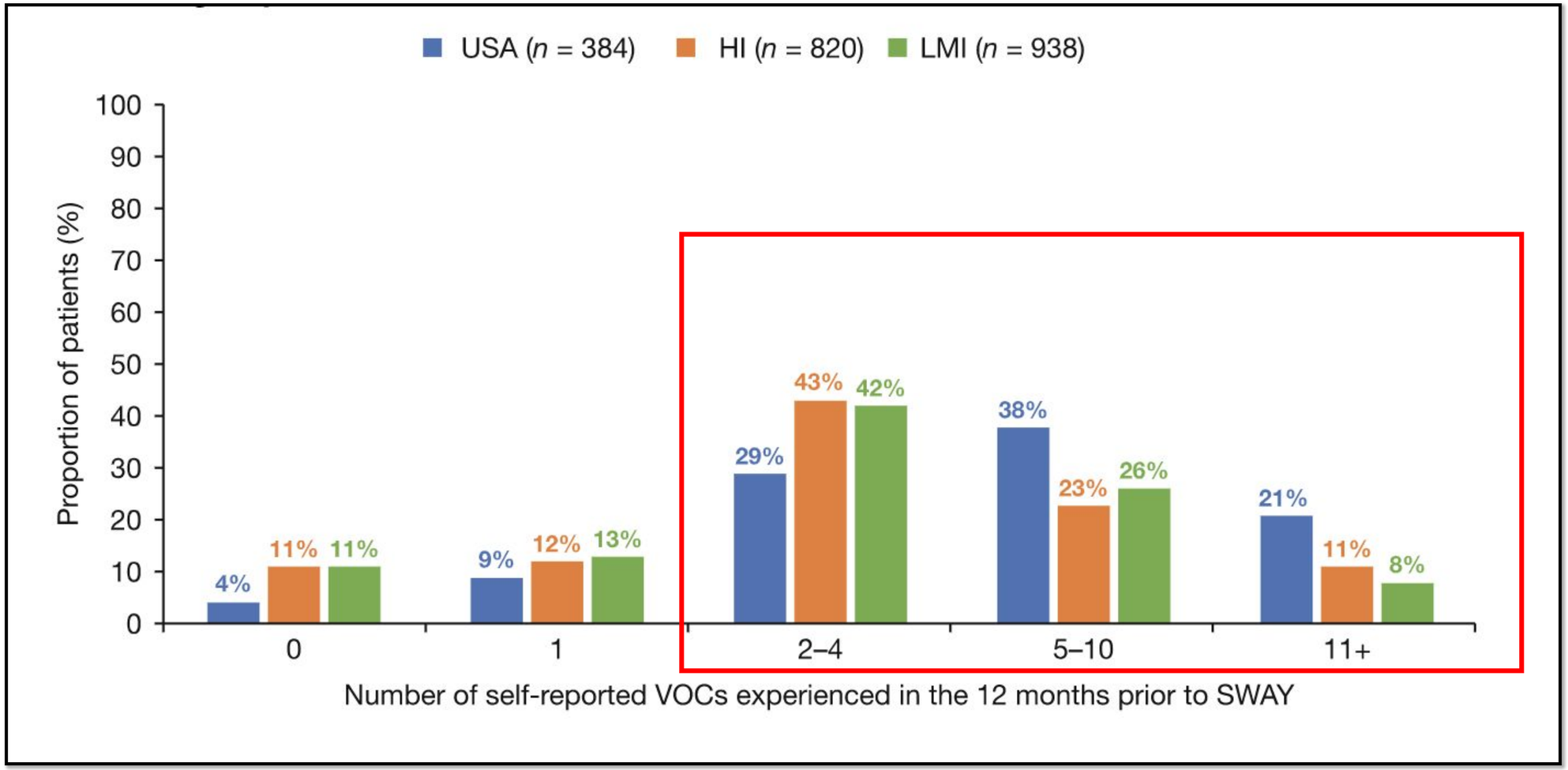
SCD survival echoes a history of “not caring” about the Black health in this country.



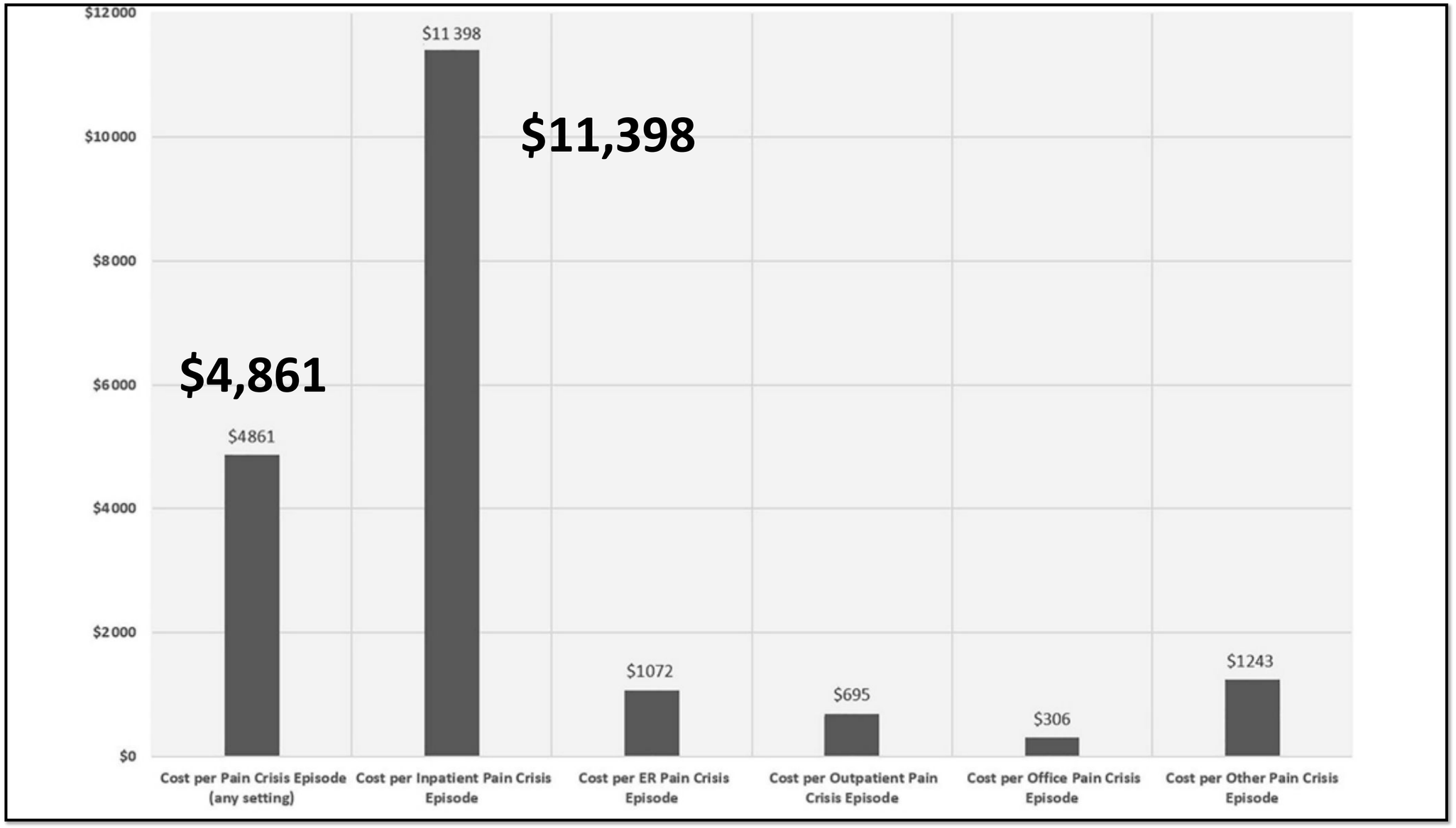
The biology of sickle cell disease is simultaneously simple and complex.



Most patients experience > 2 VOCs a year.



Healthcare costs of VOC per episode by location.



VOC has a severe impact on medical resource use and costs among the adult SCD population.

- ~ 8000 eligible adult patients
- The average total annual medical costs were $34,136 and SCD accounted for 60% of the total costs
- Patients with >3 episodes had the highest annual SCD-related costs (mean = $58,950) across all settings.
- Health care resource utilization (HCRU) and costs increased substantially as the number of VOC episodes increased.

Pain is complicated.

what everyone cares about

what patients deal with

SCD is not a disorder of pain, it is a disorder of ↓ life expectancy, accelerated aging and organ failure.

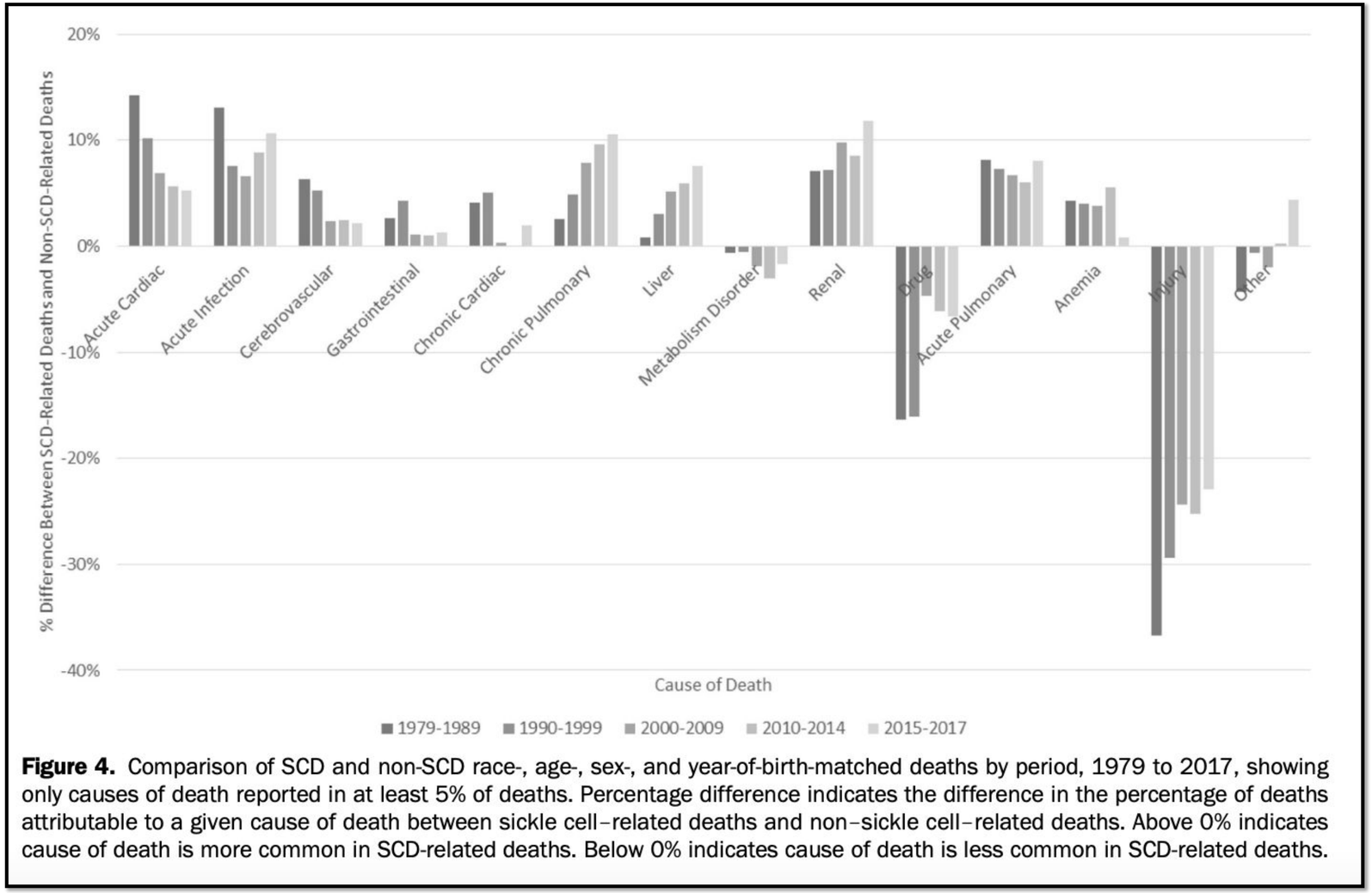
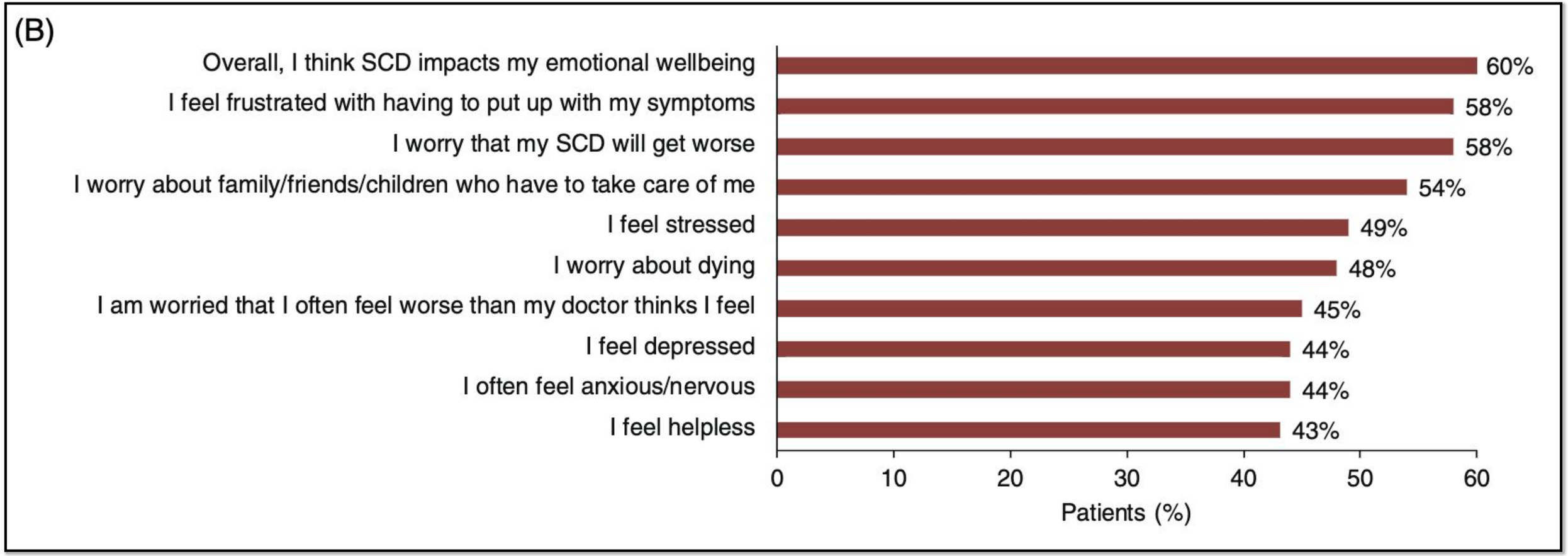


Figure 4. Comparison of SCD and non-SCD race-, age-, sex-, and year-of-birth-matched deaths by period, 1979 to 2017, showing only causes of death reported in at least 5% of deaths. Percentage difference indicates the difference in the percentage of deaths attributable to a given cause of death between sickle cell–related deaths and non–sickle cell–related deaths. Above 0% indicates cause of death is more common in SCD-related deaths. Below 0% indicates cause of death is less common in SCD-related deaths.

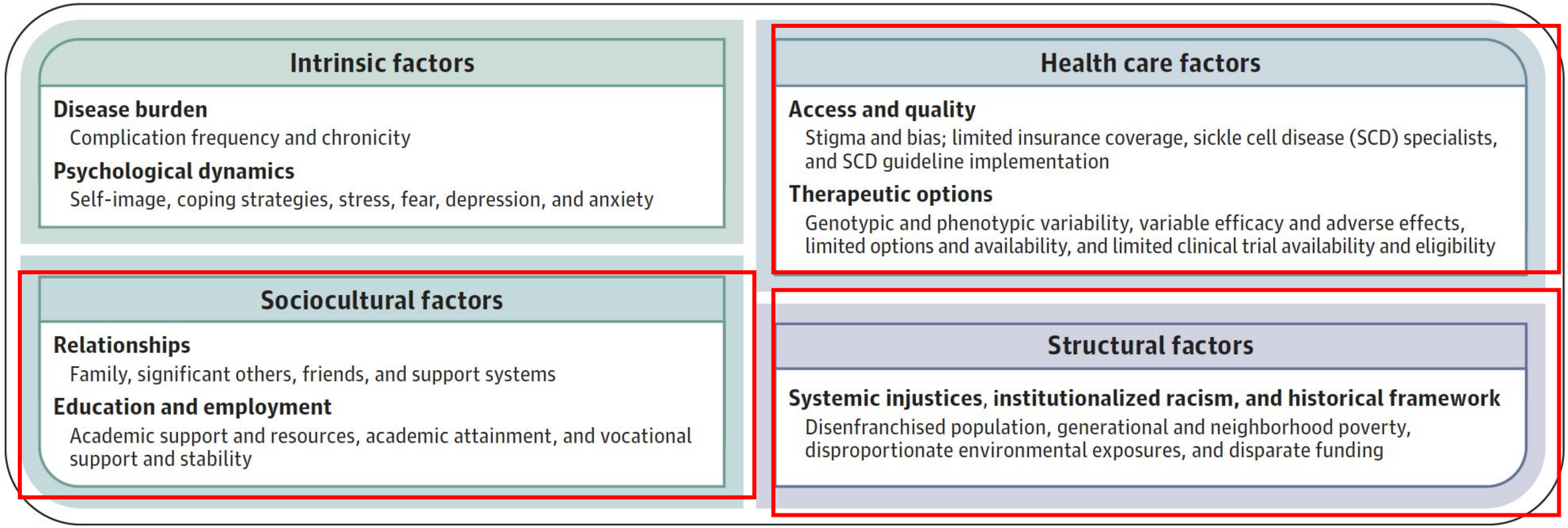
The direct economic burden of SCD is substantial.

- The lifetime care costs of SCD is ~ $1.7 million.
  - 907% more than healthy controls
- The corresponding out of pocket estimates were ~ $42,000.
  - 285% more than healthy controls
- Payers bore >95% of these costs, and the rest were paid by patients out of pocket.
- The annual OOP burden of SCD is more than 3% of the median household income of Black Americans, whose income and assets are already far below those of White Americans

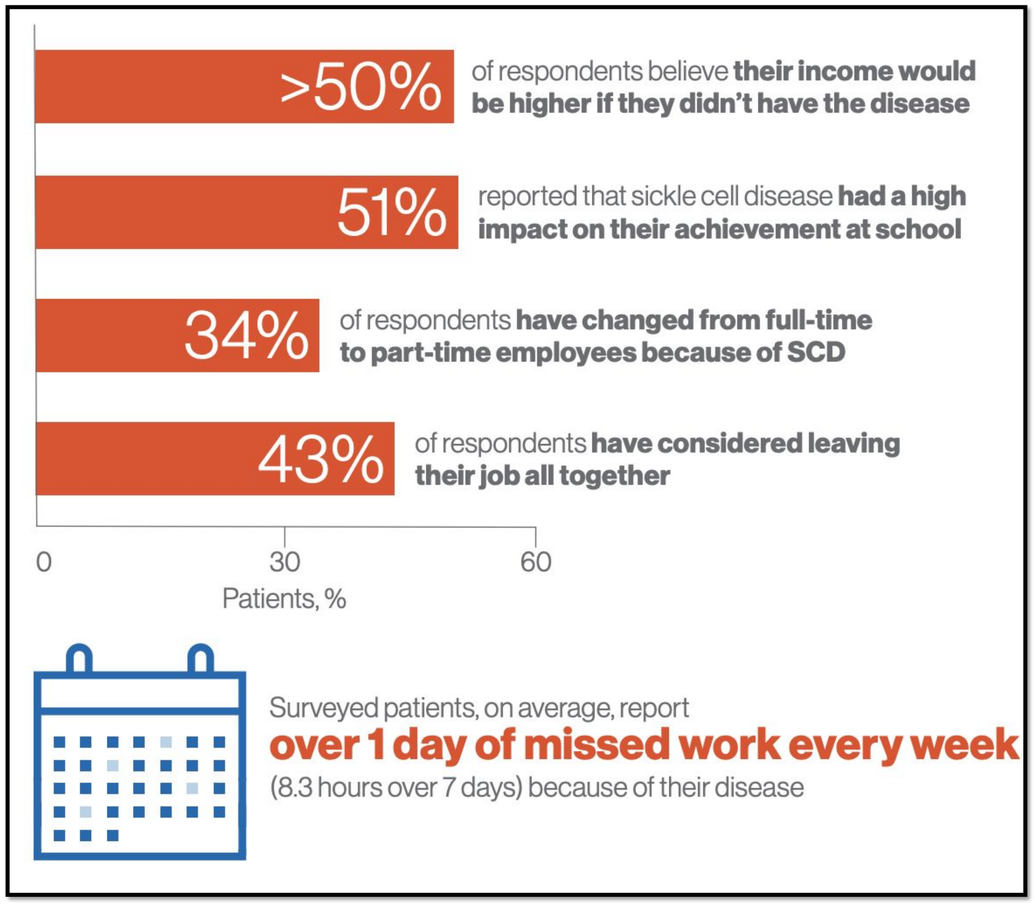
SCD is a BIOPSCYHOSOCIAL disease.

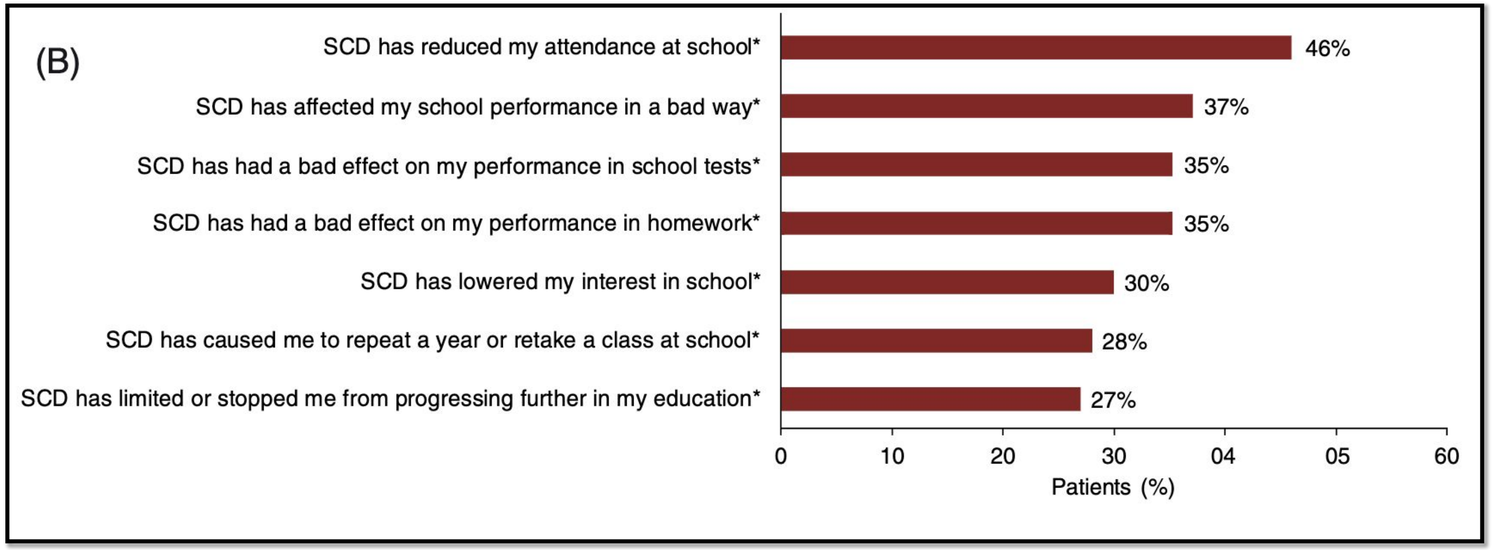


The sociocultural, structural and access/quality issues also play a major part.



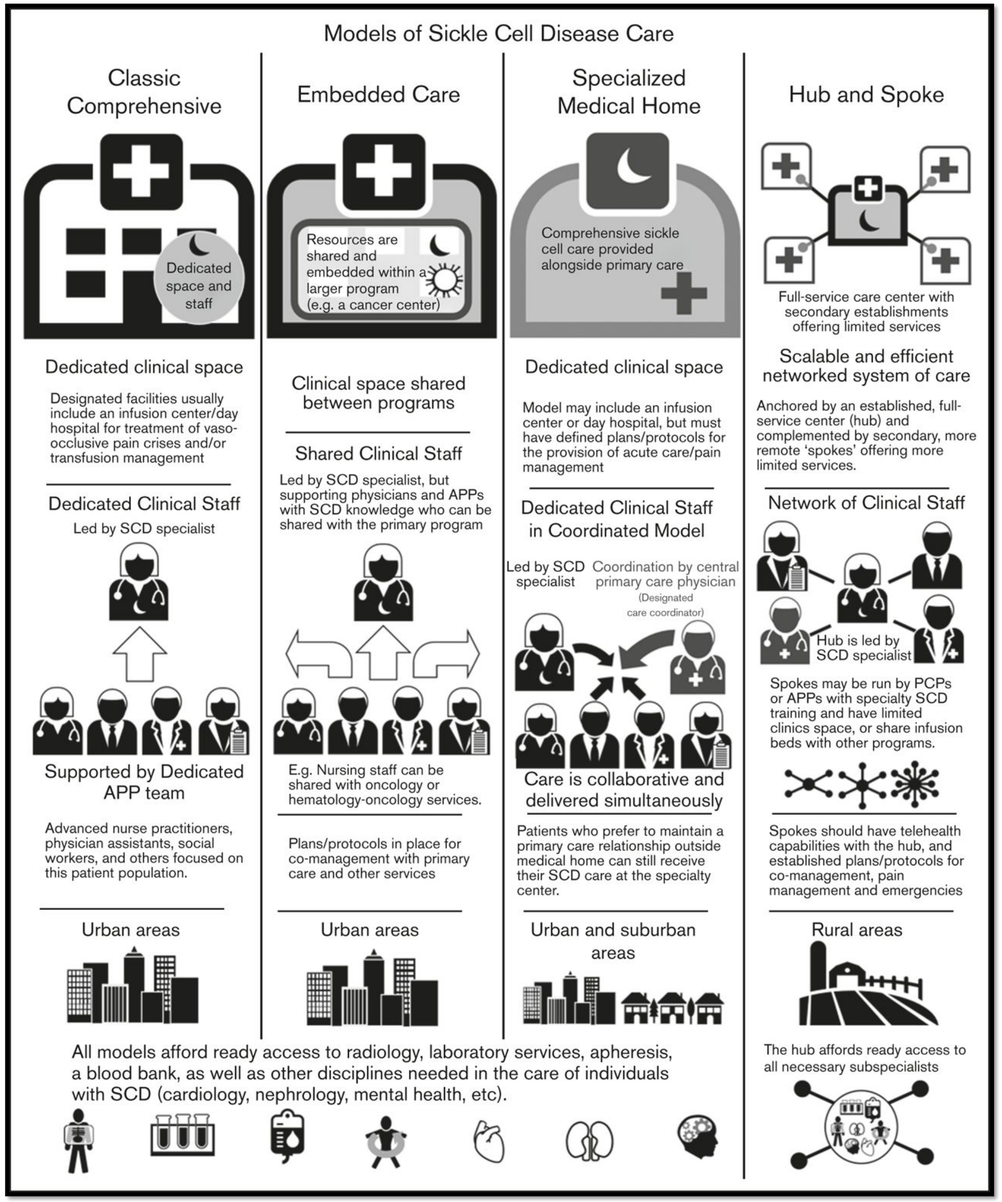
What is the cost of missed work and school?



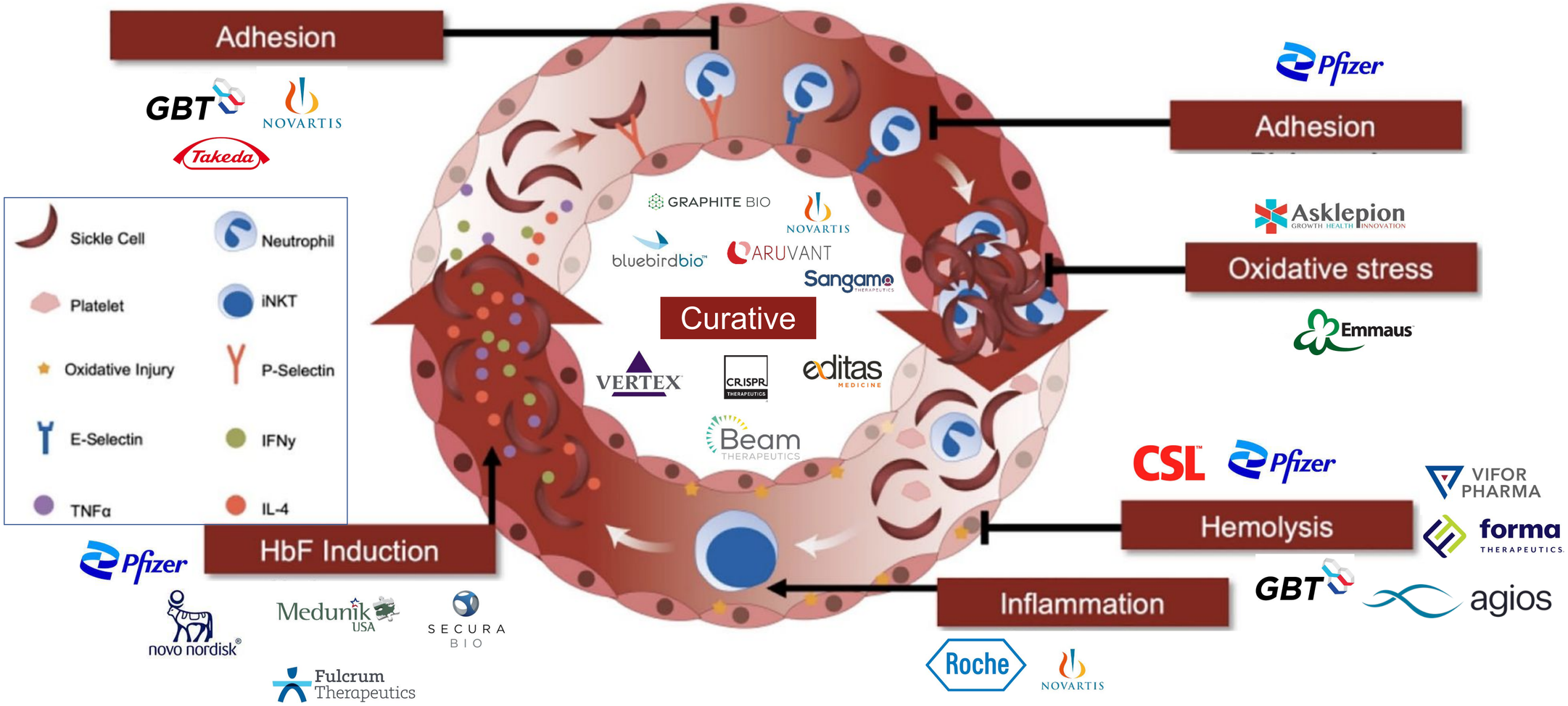


Patients with SCD are drowning.

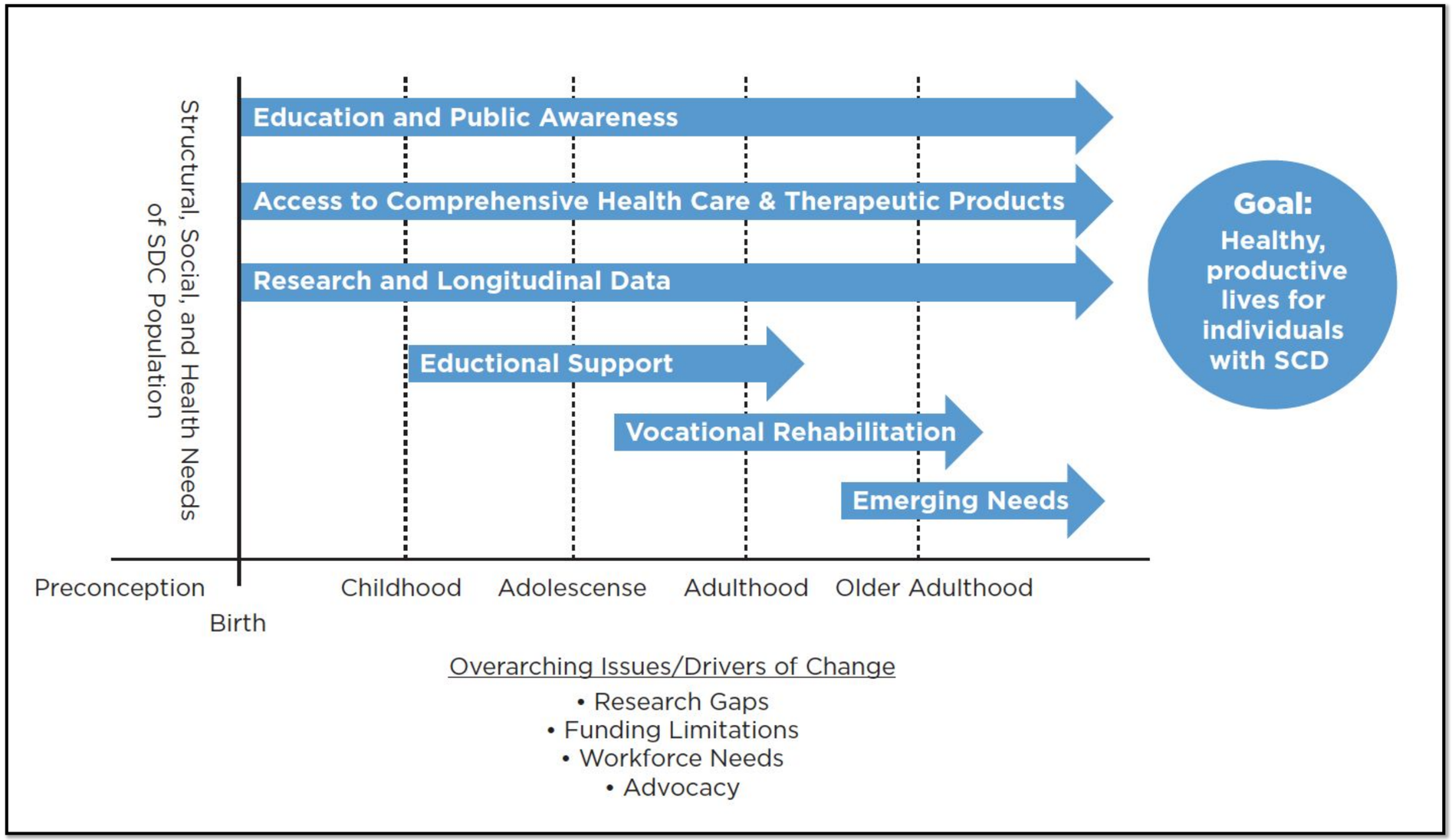
Ideas for care models, but can we fund it?



A robust pipeline, but will it get to the patients?



A blueprint to better days, but can we build it?



ubuntu

In Africa there is a concept known as 'ubuntu' - the profound sense that we are human only through the humanity of others; that if we are to accomplish anything in this world it will in equal measure be due to the work and achievement of others.

only your humanity, can re-humanize care for patients with sickle cell disease.

Q&A Session

12:25 - 12:30 PM

This Q&A Session will last for about 5 minutes.

Please take a moment to submit your questions via the "Q&A & Chat" box.

Dr. Ahmar U. Zaidi

Ashley Valentine, MRes
Sick Cells